GENE REGULATION THERAPY INVOLVING FERRITIN

ABSTRACT OF THE DISCLOSURE

A method is described for regulating gene expression related to iron metabolism to ameliorate diseases that include sickle cell disease, cancers, neurodegenerative diseases, Friedreich's ataxia and other neuromuscular disorders, and atherosclerosis. This approach is illustrated by recent findings that show that ferritin-H, an iron-binding protein that is present in cell nuclei, can repress the human β-globin gene, the gene that is mutated in sickle cell disease. Increased expression of ferritin-H or a related ferritin-family peptide, given to effected cells either as the peptide itself (or a part thereof), as an expression clone of the ferritin-H-subfamily gene, or via a gene regulator that increases expression of the ferritin-H-subfamily gene itself, prevents or ameliorates expression of the disease state in disorders where increased availability of iron is implicated in the etiology of the disease, including those named above.